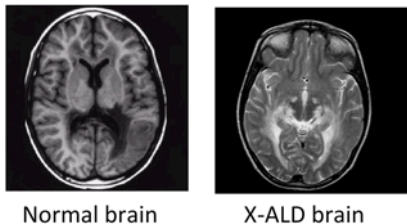




A new drug therapy and a method for the diagnosis and monitoring the progression of patients suffering from X-ALD



BUSSINESS OPORTUNITY

Available to license

IP STATUS

Patents filed and granted

TAGS

Adrenoleucodistrophy (ALD), Brain, Rare Disease, Drug Therapy, Fingolimod, Neural Demyelination

CONTACT

Valuation and Transfer Unit (UVIT) of
the University of Lleida
R&D Support Office

C/ Jaume II, 67 bis, 3rd floor
25001 – Lleida

Tel: +34 973 003 708

rdi.valoritzacio@udl.cat

MORE TECHNOLOGIES AT:
[Technology Portfolio](#)

FINGOLIMOD AS NEW DRUG THERAPY TO TREAT X-LINKED ADRENOLEUCODISTROPHY

SUMMARY

X-linked Adrenoleucodistrophy (X-ALD) is characterized by central inflammatory demyelination in the brain and/or slowly progressing axonal degeneration in the spinal cord. These neural changes are due to the loss of function of the peroxisomal transporter ABCD1 resulting in the accumulation of very-long chain fatty acids (VLCFA) that finally damage myelin surrounding nerves; this causes seizures, hyperactivity and problems for speaking, listening and understanding verbal instructions.

Fingolimod is a marketed drug for the treatment for *Multiple Sclerosis* and it has also shown effectiveness for the treatment of ALD. The drug is capable to halt immunity attack in ALD patients and to normalize levels of sphingosine 1-phosphate, thus preventing neural demyelination. Moreover, the technology includes several biomarkers/targets for the diagnosis and monitoring of this rare disease.

THE NEED MARKET

Currently there is no successful treatment for ALD, although several approaches exist:

- allogenic bone marrow transplantation (low rate of survival, a new clinical trial is in process),
- gene therapy with transfection of the ABCD2 gene (a new clinical trial is in process),
- Lorenzo's oil dietary treatment (no clinically relevant benefits),
- treatments with diverse drugs such as 4-phenylbutyrate and valproic acid (not clear results), bezafibrate (no reduction of VLCFA in either plasma or lymphocytes) or sobetirome (preliminary results).

ADVANTAGES

- › Fingolimod is a marketed drug useful for a second use in ALD.
- › No effective treatment exists for ALD.
- › Technology ready for clinical trials at Phase IIb.
- › Exclusive biomarker for the development of new products or services facing diagnosis and progress monitoring.

APPLICATIONS

Fingolimod can be used as new therapy for X-ALD patients. A specific metabolite can be used to track the progression of ALD.

LEVEL OF DEVELOPMENT

Technology demonstrated in relevant environment (TLR6) and ready for Phase II-B clinical trials.